Maryland
DEPARTMENT OF HEALTH

Orfadin (nitisinone)

Orfadin is a hydroxy-phenylpyruvate dioxygenase inhibitor indicated for the treatment of adult and pediatric patients with hereditary tyrosinemia type 1 (HT-1) in combination with dietary restriction of tyrosine and phenylalanine.

I. Criteria for Initial Approval

Orfadin will be considered for coverage when **ALL** of the criteria below are met, confirmed with supporting medical documentation.

- Documentation of a diagnosis of hereditary tyrosinemia type 1 (HT-1) with ONE of the following:
  - Diagnosis of hereditary tyrosinemia type 1 (HT-1) confirmed by biochemical testing or DNA testing.
  - Detection of succinylacetone in urine: Elevated urinary or plasma succinylacetone (SA) levels.
  - DNA testing: Mutation in the fumarylacetoacetate hydrolase (FAH) gene.
- Documentation that Orfadin is being used as an adjunct to diet modification.
- Clinical evidence or medical record documenting the use of Nityr will be ineffective or cause an adverse reaction to the patient.
- Prescribed by, or in consultation with, an endocrinologist or a metabolic or genetic disease specialist familiar with the treatment of hereditary tyrosinemia type 1 (HT-1).
- Baseline CBC, liver evaluation (including labs/imaging), Succinylacteone (SA) level, ophthalmologic testing, and body weight assessment:
  - CBC to include - Platelet and white blood cell counts (regularly during therapy).
  - Ophthalmologic examination and hepatic imaging (magnetic resonance imaging is preferred) should be performed annually.
  - Ophthalmologic examination must include slit-lamp examination prior to initiation of therapy and in those who develop symptoms of ocular toxicity.
- Plasma tyrosine (as clinically indicated with side effects; concentrations should be kept <500 micromole/L to avoid toxicity).

II. Criteria for Continuation of Therapy

All of the criteria for initial therapy (**in Section I.**) must be met; **AND**

- Provider must attest to a positive clinical response.
- Patient shows evidence of positive clinical response (e.g. decrease in urinary/plasma succinylacetone and alpha-1-microglobulin levels) while on Orfadin therapy.

EXCEPTION: If the nitisinone level is between the recommended range (40 and 60 µmol/L), monitoring plasma/urine succinylacetone may not be considered necessary.

III. Dosing/Administration

Orfadin must be administered according to the current FDA labeling guidelines for dosage and timing.

IV. Length of Authorization for Initial Therapy

Orfadin will be authorized for 12 months when criteria for initial approval are met. Continuing therapy with Orfadin will be authorized for 12 months.

V. Billing Code/Information

J8499 Prescription drug, oral, non-chemotherapeutic, not otherwise specified.

Prior authorization of benefits is not the practice of medicine nor the substitute for the independent medical judgment of a treating medical provider. The materials provided are a component used to assist in making coverage decisions and administering benefits. Prior authorization does not constitute a contract or guarantee regarding member eligibility or payment. Prior authorization criteria are established based on a collaborative effort using input from the current medical literature and based on evidence available at the time.

Approved by MDH Clinical Criteria Committee: 1/27/2021
Last Reviewed Date: 1/27/2021